Orphan Drug Policy: The Irish Experience

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What is an Orphan Medicine?

To qualify for orphan designation, a medicine must meet one of these criteria:

- Intended for diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people in the EU at the time of submission of the designation application.

- Without incentives, it is unlikely that the revenue after marketing of the medicinal product would cover the investment in its development.

- There must also be either no satisfactory method of diagnosis, prevention or treatment of the condition concerned, or, if such a method does exist, the medicine must be of significant benefit to those affected by the condition.

An Irish Solution:
Evaluating Orphan Drugs and Technologies

- The system which currently exists for evaluating new orphan drugs and technologies is the same as that for any other drug/technology. The proposal for Ireland would be that we develop a second gateway for those drugs/therapies that do not fit the main criteria in the current system.

- There are examples of innovative methods and good practice in assessing and evaluating orphan drugs and technologies both in Europe and beyond.

- However, Ireland has a unique healthcare system and cannot copy a process which works in the UK, Canada, Denmark, France or Australia.

- The health structures informing those processes in these countries are different to those that inform Irish processes. However, we can learn from other jurisdictions and put them to best use in Ireland.

Pricing and Reimbursement of Medicines

- As in all countries, Irish decision makers are challenged with the responsibility to match what appear to be infinite needs or demands with finite resources.
- Within a fixed health budget, each unit of resource can only be consumed once, and decisions to fund (or not to fund) are two sides of the one issue.
- Policymakers and decision makers are charged with responsibility for ensuring that fair decision making processes exist, which properly balance competing needs, recognising the consequences for those services and also the opportunity cost.

Who Are the Decision Makers?

- Up to May 2013, HSE Directorate of Clinical Strategy & Programmes, where applications are accepted by the HSE Corporate Pharmaceutical Unit and HTAs are carried out by the National Centre for Pharmacoeconomics.

- Recommendations in relation to pricing and reimbursement are made by the HSE Corporate Pharmaceutical Unit to the Clinical Strategy & Programmes Directorate, which is authorised to approve recommendations with minor (or no) budget impacts.

- Products with budget impacts are referred to the HSE Senior Management Team.

- Recognition that some orphan medicines are costly and that development costs can be considerable, irrespective of the number of patients to be treated.

- For an individual hospital, the financial impact of reimbursing a high-cost orphan treatment can be impossible. Need to find long-term sustainable resourcing.

Patient advocates have suggested that industry and funders need to negotiate and find innovative ways to get lifesaving treatments to patients. The cost of doing nothing is considerable.

The System in Ireland

- Until recently, access to orphan drugs was a non-issue. Pathway to reimbursement clear for all products provided under Community Drug Schemes. For hospital drugs, issues arise in that there is no national fund.
- Fundamental difficulty: hospital funding decisions are made on a singular basis, as they arise, not as part of a service plan.
- No separate pathway for access to new orphan drug treatments in Ireland.
- Clinicians and patients are reporting difficulties in finding budgetary solutions for treatments which fall outside the “normal” system.
- The pricing follows the same economic logic as drug pricing in general: High treatment costs coupled with uncertainty around magnitude or extent of treatment benefit make decisions very difficult.
- Our understanding through research suggests the Irish public wish that consideration of the rarity of a disease be included as part of the decision making process around funding of services.
- The pathway should have a robust governance structure, where nationally agreed criteria are used to make decision making transparent to all, in particular to patients.

Situation Analysis for Ireland: Legislative and Policy Statements

There is little legislative direction to guide decision makers on pricing and reimbursement of medicines in Ireland. The 1970 Health Act described categories of persons and the requirements for health boards to make arrangements for the supply of drugs, medicines and medical and surgical appliances. It did not describe how decisions relating to the designation of items would be made.

The Health Act of 2004, which established the HSE, laid down general requirements in relation to how it managed resources but did not provide any specific direction on pricing and reimbursement:

1.It defined the objective of the HSE - to use the resources available to it in the most beneficial, effective and efficient manner to improve, promote and protect the health and welfare of the public.

2.The HSE in performing its functions have regard to the resources, wherever originating, that are available to it and required it to secure the most beneficial, effective and efficient use of those resources.

3.Chief Executive Officer designated as the accounting officer of the HSE.

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•Negotiations with state and sections of the pharmaceutical industry since the 1970's influenced how applications are considered. The 2006 iteration allowed for use of Health Technology Assessment to inform pricing and reimbursement decisions and described timelines to be followed in assessments and decision making.

•For products expected to be delivered under the Community Drug Schemes (for which there is a single fund holder, i.e. the Primary Care Reimbursement Service), decisions at a national level are made in relation to both pricing and reimbursement. Once a decision is made, it is funded.

•Nationally, hospital funding decisions are made on an annual basis as part of a service plan process. Resulting in products expected to be delivered through hospitals, the national assessment process only made decisions in relation to national maximum pricing but individual hospitals / regions were responsible for submitting business cases for funding as part of the national service planning process.

•Plans to introduce Universal Healthcare will impact on the National Orphan Drugs Bill. The ethics will need to be considered for rare disease patients, particularly ultra rare.

•There is currently no separate national pricing or reimbursement application process for orphan medicines.

Strengths for Access to Orphan Drugs and Technologies in Ireland

- There are patients receiving treatment for rare diseases in the Irish system and will continue to do so.

- There are few treatments for any condition which cure disease but many ensure our patients regain a good quality of life and contribute to society

- There is consensus, developed over years by IPPOSI, MRCG and GRDO, that a system for OD's needs to be developed where societal considerations are captured in evaluations. Some rare diseases are included on the long-term illness scheme, e.g. cystic fibrosis.

Weaknesses for Access to Orphan Drugs and Technologies in Ireland

- Funding for OMPs needs to be ring-fenced,.
- New mechanisms and approaches to drug assessment need to be exploited.
- National/regional delays hamper equitable access in placing them on the market, often far beyond the legal timeframe of 180 days and due to the lack of reimbursement budget for OD.
- In Ireland, there can be inequitable access to a particular OD depending on the geographical area. A national budget is required in order to provide standardised equitable access to Irish patients.
- Industry need to interact much earlier in the development and launch cycle with policy-makers/funders. The system and practice of early interaction with the National Centre for Pharmacoeconomics (NCPE) has proven helpful in the planning process.
- The process for assessing, approving and reimbursement decisions for OD must be transparent. Decision makers need to be part of a system where patients are consulted and where there is a right to appeal decisions made with regard to access.
- Rarity, disease severity, the availability of alternative therapies need to inform decision making.

Irish Stakeholder View

- Feedback from IPPOSI consultations suggest that the existing process is not appropriate for all orphan drugs where there may be imbalance between the cost of production and the potential market size due to the rarity of a particular condition.

- A decision to treat based solely on the mathematics of health economics is not in the interest of patients with rare conditions.

- Health economic thresholds are a guide rather than an absolute, and that cognisance of clinical effectiveness, social and ethical considerations make it possible to provide many interventions. However, the process for such considerations is vague and inconsistent.

- The pathway for access to other medical interventions (medical devices) fundamental to the treatment of particular rare conditions can be unclear and difficult for the patient to access. The long-term illness scheme does not account for many rare diseases, and attaining medical cards for RD patients can be difficult.

What We Have Proposed as Advocates

- Establish early dialogue between companies and HSE.
- Passing the Health Information Bill is essential.
- Reimbursement systems need to be shaped by strong policy direction and societal guidance.
- Need for transparency so that all parties can understand the drivers of reimbursement decisions for rare diseases and timelines.
- A patient centric approach, including complex considerations governing health gain, societal value, ethics and best practice.
- Health Technology Assessment is just one element. More transparency as to pricing rationale would be welcomed by funders. The CAVOD at EU level will be an important step in this context.
- Compassionate use and temporary approval of orphan drugs: Immediate measures needed for those who cannot wait for treatments to get a license.

Reilly announces major reorganisation of acute hospitals

Move to six largely autonomous groups aims to improve outcomes for patients

May 2013:
The Irish Health Ministry Disbanded the HSE

Our health system will now be run by six separate health boards.

Unique System

There is no one country which can provide a clear synergy with the Irish Healthcare System.

The Irish system is unique and requires an Irish solution to a clear path for orphan drugs.

Who We Work with at Home and Abroad

Irish Platform for Patient Organisations Sceince & Industry – IPPOSI
www.ipposi.ie

Genetic & Rare Disorders Organisation – GRDO
www.grdo.ie

Medical Research Charities Group – MRCG
www.mrcg.ie

European Organisation for Rare Diseases – EURORDIS
www.eurordis.org

European Patients Forum – EPF
www.eu-patient.eu

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